Rocket Pharmaceuticals Announces FDA Clearance of IND Application for RP-L102 Gene Therapy for Fanconi Anemia

November 7, 2018

- Patients to Be Treated with RP-L102 under "Process B" Incorporating Higher Cell Doses, Transduction Enhancers, and Commercial-grade Vector Manufacturing and Cell Processing –

- U.S. Trial to Commence Early 2019; No Conditioning Required -

- Center for Definitive and Curative Medicine at Stanford University School of Medicine to Lead U.S. Clinical Studies -

NEW YORK--(BUSINESS WIRE)--Nov. 7, 2018-- Rocket Pharmaceuticals, Inc. (Nasdaq: RCKT) ("Rocket"), a leading U.S.-based multi-platform gene therapy company, announces the clearance of the Company's Investigational New Drug (IND) application for RP-L102, the Company's lentiviral vector (LVV)-based gene therapy for the treatment of Fanconi Anemia (FA), by the U.S. Food and Drug Administration (FDA). The clinical trial will evaluate "Process B" which incorporates higher cell doses, transduction enhancers, and commercial-grade vector manufacturing and cell processing. This process improves upon the first-generation process developed in partnership with the Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT))/Ciber of Rare Diseases/Fundación Jiménez Díaz in Madrid, Spain.

"Today marks Rocket's first IND clearance by the FDA and represents an important step forward for the patients and families suffering from FA. Our team worked expeditiously to file this application ahead of schedule so that we may bring RP-L102 forward as quickly as possible," said Kinnari Patel, Pharm.D., MBA, Chief Operating Officer and Head of Development of Rocket. "The U.S. clinical trial will begin in early 2019. The goal of the trial is to evaluate the safety, tolerability, and efficacy of 'Process B' RP-L102 in patients with FA. No conditioning is required due to the selective advantage that is present in FA. We look forward to engaging with regulatory authorities on a final registration path in the second half of 2019 after initial patients have been treated."

The planned clinical trial of "Process B" RP-L102 is expected to enroll approximately 12 FA patients at the Center for Definitive and Curative Medicine at Stanford University School of Medicine, Hospital Niño Jesús/CIEMAT, and other leading centers in the U.S. and in the EU.

About RP-L102 (LVV-based gene therapy for Fanconi Anemia)

RP-L102 is Rocket's lentiviral vector (LVV)-based gene therapy in development for patients with FA with Rocket's collaboration partners at Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT) in Spain, CIBER-Rare Diseases and IIS-Fundación Jiménez Díaz. The International Fanconi Anemia Gene Therapy Working Group helped the development of new generation of FA gene therapy programs, which began with a HIV-1-derived, self-inactivating lentiviral vector. RP-L102's lentiviral vector carries the FANC-A gene as part of the PGK-FANCA-WPRE expression cassette which includes a phosphoglycerate kinase (PKG) promoter and an optimized woodchuck hepatitis virus posttranscriptional regulatory element (WPRE). The ex vivo administration process begins with the removal and isolation of hematopoietic stem cells using a CD34+ selection process. Autologous genetically modified CD34+ enriched hematopoietic cells (fresh or cryopreserved) are infused back into patients to restore function. RP-L102 is currently being studied in a Phase 1/2 clinical trial in the European Union with an Investigational Medicinal Product Dossier (IMPD) in place with the Spanish Agency for Medicines and Health Products. The U.S. Food and Administration accepted the Company's Investigational New Drug (IND) application for RP-L102 utilizing "Process B" which incorporates higher cell doses, transduction enhancers, and commercial-grade vector. RP-L102 has been granted Orphan Drug designation for the treatment of Fanconi Anemia type A in the United States and in Europe.

About Fanconi Anemia

Fanconi Anemia (FA) is a rare pediatric disease characterized by bone marrow failure, malformations and cancer predisposition. The primary cause of death among patients with FA is bone marrow failure, which typically occurs during the first decade of life. Allogeneic hematopoietic stem cell transplantation (HSCT), when available, corrects the hematologic component of FA, but requires myeloablative conditioning, which is highly toxic for the patient HSCT is frequently complicated by graft versus host disease and also increases the risk of solid tumors, mainly squamous cell carcinomas. Approximately 60-70% of patients with FA have a FANC-A gene mutation, which encodes for a protein essential for DNA repair. Mutation in the FANC-A gene leads to chromosomal breakage and increased sensitivity to oxidative and environmental stress. Chromosome fragility induced by DNA-alkylating agents such as mitomycin-C (MMC) or diepoxybutane (DEB) is the 'gold standard' test for FA diagnosis. The DEB assay can further differentiate FA patients from somatic mosaic patients. Somatic mosaicism occurs when there is a spontaneous reversion mutation that can lead to a

mixed chimerism of corrected and uncorrected bone marrow cells leading to stabilization or correction of an FA patient's blood counts in the absence of any administered therapy. Somatic mosaicism provides strong rationale for the development of FA gene therapy and demonstrates the selective advantage of gene-corrected hematopoietic cells in FA[1].

[1]Soulier, J.,et al. (2005) Detection of somatic mosaicism and classification of Fanconi anemia patients by analysis of the FA/BRCA pathway. Blood 105: 1329-1336

About Rocket Pharmaceuticals, Inc.

Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) ("Rocket") is an emerging, clinical-stage biotechnology company focused on developing first-in-class gene therapy treatment options for rare, devastating diseases. Rocket's multi-platform development approach applies the well-established lentiviral vector (LVV) and adeno-associated viral vector (AAV) gene therapy platforms. Rocket's lead clinical program is a LVV-based gene therapy for the treatment of Fanconi Anemia (FA), a difficult to treat genetic disease that leads to bone marrow failure and potentially cancer. Preclinical studies of additional bone marrow-derived disorders are ongoing and target Pyruvate Kinase Deficiency (PKD), Leukocyte Adhesion Deficiency-I (LAD-I) and Infantile Malignant Osteopetrosis (IMO). Rocket is also developing an AAV-based gene therapy program for an undisclosed rare pediatric disease. For more information about Rocket, please visit www.rocketpharma.com.

Rocket Cautionary Statement Regarding Forward-Looking Statements

Various statements in this release concerning Rocket's future expectations, plans and prospects, including without limitation, Rocket's expectations regarding the safety, effectiveness and timing of product candidates that Rocket may develop, including in collaboration with academic partners, to treat Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD) and Infantile Malignant Osteopetrosis (IMO), and the safety, effectiveness and timing of related pre-clinical studies and clinical trials, may constitute forward-looking statements for the purposes of the safe harbor provisions under the Private Securities Litigation Reform Act of 1995 and other federal securities laws and are subject to substantial risks, uncertainties and assumptions. You should not place reliance on these forward-looking statements, which often include words such as "believe", "expect", "anticipate", "intend", "plan", "will give", "estimate", "seek", "will", "may", "suggest" or similar terms, variations of such terms or the negative of those terms. Although Rocket believes that the expectations reflected in the forward-looking statements are reasonable, Rocket cannot guarantee such outcomes. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including, without limitation, Rocket's ability to successfully demonstrate the efficacy and safety of such products and pre-clinical studies and clinical trials, its gene therapy programs, the preclinical and clinical results for its product candidates, which may not support further development and marketing approval, Rocket's ability to commence a registrational study in FA within the projected time periods, the potential advantages of Rocket's product candidates, actions of regulatory agencies, which may affect the initiation, timing and progress of pre-clinical studies and clinical trials of its product candidates, Rocket's and its licensors ability to obtain, maintain and protect its and their respective intellectual property, the timing, cost or other aspects of a potential commercial launch of Rocket's product candidates, Rocket's ability to manage operating expenses, Rocket's ability to obtain additional funding to support its business activities and establish and maintain strategic business alliances and new business initiatives, Rocket's dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, the outcome of litigation, and unexpected expenditures, as well as those risks more fully discussed in the section entitled "Risk Factors" in Rocket's Annual Report on Form 10-K for the year ended December 31, 2017. Accordingly, you should not place undue reliance on these forward-looking statements. All such statements speak only as of the date made, and Rocket undertakes no obligation to update or revise publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

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Source: Rocket Pharmaceuticals, Inc.

Claudine Prowse, Ph.D.
SVP, Corporate Strategy and IRO
Rocket Pharma, Inc.
The Empire State Building, Suite 7530
New York, NY 10118
cp@rocketpharma.com
www.rocketpharma.com
investors@rocketpharma.com